NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

Statement to the Joint Meeting of the Transmissible Spongiform Encephalopathies Advisory Committee and the Blood Products Advisory Committee from the National Hemophilia Foundation

The National Hemophilia Foundation (NHF) would like to take the opportunity to provide written comments to the joint meeting of the Transmissible Spongiform Encephalopathies Advisory Committee and the Blood Products Advisory Committee on the revised *FDA Guidance on Preventive Measures to Reduce the Possible Risk of Transmission of Creutzfeldt-Jakob Disease (CJD) and Variant Creutzfeldt-Jakob Disease (vCJD) by Blood and Blood Products.* NHF is a not-for-profit organization dedicated to improving the quality of life for all individuals with hemophilia and other bleeding disorders.

NHF commends the Food and Drug Administration (FDA), the Centers for Disease Control and Prevention (CDC), the National Institutes of Health, other agencies within the Public Health Service, and the Department of Agriculture for the high level of importance it has placed on preventing an outbreak of bovine spongiform encephalopathy (BSE) and vCJD in the United States. The federal government has taken an aggressive, multi-pronged approach toward preventing an outbreak of BSE and vCJD in the United States. While NHF supports the on-going efforts of the FDA and other federal agencies to protect the American public from the possible risk of transmission of vCJD through human blood or plasma, NHF remains concerned that the most recent guidance document, once again, leaves us vulnerable to an avenue of transmission by failing to require deferral of source plasma from individuals who meet the donor deferral criteria for whole blood.

CJD is a rare but universally fatal condition associated with a poorly understood transmissible agent that may arise from genetic mutations in the prion proteins. vCJD, a related disorder, was shown to be related to a CJD-like infection in cattle and other ruminants. The mode of transmission and the factors that affect disease susceptibility have not been fully defined and therefore remain highly imprecise.

In October 2000, millions of British television viewers experienced the final and brutal last days of 14-year-old Zoe Jeffries of Manchester, England. Zoe's tragic and painful death from vCJD was chronicled over two years. The hemophilia community shares in the grief of the Jeffries family, a grief with which we are most familiar.

In January 1983, the Medical and Scientific Advisory Council (MASAC) of NHF was convened in New York City to develop a position statement on AIDS in relation to

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blood product procurement and use in hemophilia. At that time, MASAC reviewed the most recent scientific data available and heard testimony from eminent scientists and officials from the FDA and the CDC that had earlier that month participated in a conference on the risks associated with AIDS transmission through blood and blood products. At that time, the experts assured our community that they had little proof that individuals with hemophilia would contract HIV through the use of clotting factor products, and because of their expert advice and counsel MASAC issued a series of twelve treatment recommendations that encouraged patients with hemophilia to continue to treat their disorder with what we now know were contaminated blood products.

The results of those actions were catastrophic and continue to haunt this community and NHF to this very day. As a result of the FDA, CDC and MASAC recommendations, nearly 9,000 individuals with hemophilia and other bleeding disorders received contaminated blood products between 1978 and 1985. In turn, these individuals infected nearly one thousand spouses, partners, and children, leading to the untimely deaths of thousands of individuals and in some cases of entire families. Biomedical research has provided us with some respite from the ravages of this tragic disease. The development of protease inhibitors and other treatment advances have provided hope and allowed many individuals with HIV to live somewhat normal lives.

This tragic story is far from over. Individuals with hemophilia and other bleeding disorders have also been infected with various strains of hepatitis, parvovirus and other pathogens as a result of the use of contaminated blood products. An estimated 75% of all individuals with hemophilia over the age of twelve have chronic hepatitis C (HCV) infection, and approximately one-third of this group are co-infected with HIV. In the United States and Europe, the prevalence of hepatitis A among individuals with hemophilia, ranges from 40%-67%.

The continued presence of men, women, and children in our community with HIV, HCV and other infections serve as a powerful reminder that we must not lose our resolve and that vigilance must be our watchword – we will not and cannot allow this community to be placed at risk again. Unfortunately, the guidance document reissued on January 9, 2002, does just that.

The revised preventive measures to reduce the possible risk of transmission of CJD and vCJD by blood and blood products contain eight criteria for donor deferral. NHF generally concurs with criteria one through seven, but questions the failure of the FDA to apply the same deferral criteria for travel to countries in Asia where cases of bovine spongiform encephalopathy have been recorded. NHF has serious reservations with criterion eight, which would allow individuals who would meet the criteria for deferral for whole blood, blood components for transfusion and source leukocytes to continue to remain eligible for source plasma donation.

Worldwide, over 85 brands of clotting factor concentrates currently exist for the treatment of congenital and acquired bleeding disorders. These concentrates are distributed by more than 27 companies in 16 countries. According to the World

Hemophilia Foundation, an exact count of brands is difficult because one fractionation plant may use the same method with the same or different plasma sources to make a given product under more than one name for various distributors or markets. In the United States, the American Red Cross distributes concentrates made from plasma recovered from unpaid whole blood donations. However, large commercial fractionators (Aventis, Bayer, Baxter and others) in this country use source plasma from paid plasmapherisis donors. In Europe, national fractionator centers produce concentrates from plasma recovered from both voluntary and paid whole blood and apheresis donations from the United States and Western Europe. It would seem that mandating one set of criteria for whole blood donation and another for source plasma donation would only serve to further complicate this already complex manufacturing sector as well as increase the risk that clotting factor derived from whole blood donations could accidentally be introduced into the U.S. market, placing individuals with hemophilia at risk for CJD and vCJD.

Throughout the guidance document, the FDA reiterates the absence of data linking the transmission of CJD and vCJD infection through blood transfusion and the use of blood products. While it is true that no causative link has been established, it is equally true that the scientific community has no irrefutable data that demonstrates that infection cannot occur through the use of blood and blood products. To date, there is no FDA-approved test to determine the presence of CJD and vCJD infections in blood and plasma donors. While plasma derivatives are highly processed materials, unlike whole blood, the absence of a reliable test for CJD and vCJD calls into question the FDA's assumptions that the plasma fractionation process may reduce infectivity and therefore donations for source plasma are inherently safer.

That the FDA will continue to evaluate this document in light of "evolving experimental and epidemiological information" is encouraging. Therefore, we would urge you to err on the side of caution and to include donation for source plasma in the CJD/vCJD deferral criteria until such time as science provides us with definitive answers in this area. We fully realize that this request raises concerns about the availability of an adequate number of donors. However, until we can definitively tell our families that medical technologies exist to guarantee their safety from CJD and vCJD, our answer must be a pledge to work to increase the overall number of blood and apheresis donors and to cultivate these individuals to become long-term donors. The safety of our community must remain our primary focus.

We thank you for this opportunity to address you once again on this matter.